

NephCure Accelerating Cures Institute: A Multidisciplinary Consortium to Improve Care for Nephrotic Syndrome



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Introduction: NephCure Accelerating Cures Institute (NACI) is a collaborative organization sponsored by NephCure Kidney International and the University of Michigan. The Institute is composed of 7 cores designed to improve treatment options and outcomes for patients with glomerular disease: Clinical Trials Network, Data Warehouse, Patient-Reported Outcomes (PRO) and Endpoints Consortium, Clinical Trials Consulting Team, Quality Initiatives, Education and Engagement, and Data Coordinating Center.

Methods: The Trials Network includes 22 community- and hospital-based nephrology practices, 14 of which are trial-only sites. Eight sites participate in the NACI Registry, and as of October 2017, 1054 patients are enrolled with diagnoses including but not limited to focal segmental glomerulosclerosis, minimal change disease, membranous nephropathy, IgA nephropathy, and childhood-onset nephrotic syndrome. By using electronic health record data extraction, robust and efficient clinical data are captured while minimizing the burden to site-based network staff.

Results: The Data Warehouse includes her-extracted data from registry patients, PRO development data, and data from completed observational studies and clinical trials. The Clinical Trial Consulting Team provides support for trial design in rare diseases leveraging these data. The PRO and Endpoints Consortium develops shorter-term endpoints while capturing the patient-reported significance of interventions under study. The Quality Initiatives and Education/Engagement cores elevate the level of care for patients. The Data Coordinating Center manages the analysis and operations of the Institute.

Conclusion: By engaging with patients, academia, industry, and patient advocate community representatives, including our Patient Advisory Board, NACI strives for better outcomes and treatments using evidence-based support for clinical trial design.

Kidney Int Rep (2018) 3, 439–446; <https://doi.org/10.1016/j.ekir.2017.11.016>

KEYWORDS: clinical trials; electronic health record; glomerular disease; nephrotic syndrome; patient advisory; patient-reported outcomes

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Received 3 October 2017; revised 14 November 2017; accepted 21 November 2017; published online 28 November 2017

In 2009, *Kidney Disease: Improving Global Outcomes Glomerulonephritis Guidelines* were published and included recommendations for the management of primary proteinuric glomerular diseases, including focal segmental glomerulosclerosis, minimal change

disease, and membranous nephropathy, and steroid-sensitive and steroid-resistant nephrotic syndrome (NS) in children. The evidence grade for these guidelines was quite weak due to a paucity of clinical trials. In addition, commonly used therapies have adverse side effects that adversely influence drug tolerability, dose, and duration. The lack of effective and safe therapeutic options results in adverse short-, intermediate-, and long-term outcomes.

Primary proteinuric kidney diseases (PPKDs), including focal segmental glomerulosclerosis, minimal change disease, and membranous nephropathy, are rare chronic health conditions that negatively affect short-term health and often lead to end-stage kidney disease.¹ The condition of NS with edema, hypoalbuminemia, and large urinary protein losses is associated with an increased risk for acute kidney injury, thromboembolic events, serious infections, and impaired health-related quality of life.²⁻⁵ Nondiabetic glomerular diseases are leading causes of end-stage kidney disease, accounting for 6.3% of incident end-stage kidney disease in adults and 20% of end-stage kidney disease in children,⁶ and 14.3% of prevalent disease in adults and 21.8% in children.^{7,8} Taken together, PPKDs represent a significant patient and public health problem.

In the past decade, advances in biomedical research have occurred that aid in the understanding of the biologic underpinnings of NS.⁹⁻¹² Leveraging these scientific advances into viable treatments and improvement in clinical outcomes depends on our ability to perform high-quality, efficient, and successful clinical trials.^{13,14}

Over the past decade, it has become clear that a novel approach to the care of patients, development of trials, and conduct of trials is necessary to improve outcomes in patients with proteinuric kidney disease. Early and meaningful input from diverse stakeholders, including patients, advocacy groups, industry, clinicians, payers, and researchers, is likely to stimulate progress where traditional individual stakeholder approaches have failed. Recognizing the essential need for innovation, NephCure Kidney International (NKI), a non-for-profit advocacy group, and the University of Michigan partnered with clinicians, patients, and scientists to develop NephCure Accelerating Cures Institute (NACI). NACI was founded in 2015 as a unique collaborative to improve the treatment options and health outcomes of patients with glomerular disease. Barriers to the development and conduct of clinical trials for glomerular diseases and models used to address similar concerns in other health conditions have been identified and used to create the organizational framework of NACI (Figure 1).¹⁵⁻¹⁸ The purpose of this article was to share information about NACI that

may serve as a model for other rare disease therapeutic development initiatives.

METHODS

Data Management and Analysis

Key sources of data are accessible to support trial development, analysis simulation, and quality initiatives. The NACI Data Warehouse manages and stores data from the NACI patient registry, and active and completed trials. This data resource is available for hypothesis generation, hypothesis testing, clinical trial modeling, targeted enrollment, and quality improvement efforts. To support efficient data collection for consenting NACI Registry participants, the participating nephrology practices extract electronic medical records, transform the data into the NACI common data model, and submit data files from the date of electronic health record patient record initiation with monthly updates using secure data transfer methods.

The NACI Data Warehouse includes subject matter areas, including patient demographics, diagnoses, encounters, vital signs and status, clinical laboratory results, medications, procedures, kidney biopsy reports, and end-stage kidney disease and transplant-specific data for patients in the registry. The NACI Data Warehouse also allows for electronic data capture using manual data entry for domains requiring confirmation by the local clinical care team or where local electronic health records have incomplete data domains. Data visualization is provided to all participating sites for local population management and study governance.

Clinical Trials Consulting

A key component to the successful conduct and execution of clinical trials occurs at the trial development phase. During trial development, multiple stakeholders participate in trial design, feasibility, and acceptability to patients' and clinicians' assessment, and consent and recruitment material review. The Clinical Trials Consulting Core represents a feature within NACI that supports clinical trial development and review for investigator and industry-initiated studies. The consultations are tailored to the needs of the requesting investigator with options for participation in a single day workshop with experts in trial design, epidemiology, translational research, clinician and patient engagement, and operational feasibility or remote review of a developed protocol by appropriate experts.

Clinical Trials Network

The Clinical Trials Network is composed of nephrology practices in hospital and community-based settings with clinical trials expertise. The practices have a focus on children, adults, or a combination, thereby ensuring

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